



March 4, 2016

The Honorable Ron Wyden
U.S. Senator
Committee on Finance
U.S. Senate
Washington, DC 20510

The Honorable Charles Grassley
U.S. Senator
Committee on Finance
U.S. Senate
Washington, DC 20510

Dear Senator Wyden and Senator Grassley:


On behalf of America's Health Insurance Plans (AHIP), we appreciate this opportunity to offer comments to the policy questions raised in your report, "The Price of Sovaldi and Its Impact on the U.S. Health Care System," released on December 1, 2015.

The questions in your report are critical to addressing the explosive growth in prices for prescription drugs. Consumers -- as well as hospitals, providers, employers, and state Medicaid directors -- have grown increasingly frustrated by drug costs that show no sign of slowing down. We strongly believe that greater transparency around drug pricing and more competition in the market are critical to support sustainable, private-sector solutions that deliver the best value for patients and the health system. Specifically, health plans support the following:

- Encouraging alternative payment and incentive structures for new drugs and technology;
- Shortening the exclusivity period for biologics to promote competition;
- Pursuing policies that support the development of a robust biosimilar market;
- Prohibiting abuse of the patent process by drug companies;
- Expanding research on treatment effectiveness; and
- Promoting transparency on prescription drug research, development, and pricing.

We applaud your leadership in this area and are committed to working with you to address this critical issue. Our detailed responses to your questions follow.

Sincerely,


Matthew Eyles
Executive Vice President
Policy and Regulatory Affairs

1) What are the effects of a breakthrough, single source innovator drug on the marketplace?

As noted in the Committee's report, "...not until reasonable competition entered the marketplace did Gilead's pricing incentives and behavior change. Not all expensive innovator drugs face competition so soon after launch, and thus the next expensive innovator drug could potentially create significant budgetary pressures for federal payers and lead to access restrictions for an extended timeframe."

While the cost pressures from Sovaldi are well-documented, we also believe this issue extends beyond "breakthrough" drugs. Policymakers should look broadly at all high cost, single-source innovator medications and treatments. This would include drugs that may be approved through any of the FDA's four expedited pathways: fast track, breakthrough therapy designation, priority review, and/or accelerated approval. It should also include high-cost, single source drugs that were not approved through any of these pathways, as expedited approval may not have been requested by the manufacturer.

The FDA's expedited programs are intended to accelerate approval for drugs that address an unmet medical need in the treatment of a serious or life-threatening condition. Unfortunately, these goals will not be met if treatments are priced at such high levels that the cost leads to access and affordability concerns. To that end, we believe policymakers should explore how these expedited programs could be utilized to encourage additional market entrants for classes of high-cost drugs that have no competitors, or perhaps a very limited number of competitors. For example, policymakers could consider an FDA-directed initiative to grant a priority review designation to New Drug Applications (NDAs) and Biologics License Applications (BLAs) that would be a direct competitor to a single source innovator drug or biologic. The first competitor to such a drug would receive a more expeditious FDA review, potentially cutting the review period from 10-12 to six months. The earlier these competitors enter the market, the greater the opportunity for payers to negotiate discounts for consumers.

In developing policy solutions to promote competition, we also urge an examination of current market dynamics in different therapeutic areas in light of pricing trends that seem to defy basic economics. For example, in April 2015, a study was published in *Neurology* that found costs of disease-modifying therapies (DMTs) for the treatment of multiple sclerosis increased sharply despite the availability of an increased number of these treatments. Specifically, first generation DMTs range from \$8,000-\$11,000 a year, but now all DMTs – regardless of how long they have been in the market – cost upward of \$60,000 annually. As noted by the authors, "While we would expect that legitimate advances, such as the development of oral DMTs, might garner higher prices, the escalation in costs for first-generation agents that have been available for up to 2 decades is puzzling."¹

¹ Hartung, et al. "The Cost of Multiple Sclerosis Drugs in the U.S. and the Pharmaceutical Industry." *Neurology*. April 4, 2015.

We also see troubling market dynamics in the area of oncology. A new cancer drug now often costs more than \$100,000 a year. Manufacturers in the oncology space appear to look at the entire landscape of oncology treatments (regardless of specific cancer type) and benchmark the price of new therapies to the highest-priced oncology treatment current available. However, high price tags have nothing to do with the drug's level of innovation or efficacy for patients, according to a study in JAMA Oncology. In the study, researchers from the National Institutes of Health examined 51 oncology drugs approved by the FDA from 2009 through 2013 and concluded that current pricing models were not rational, which was evidenced by the fact that prices had no significant correlation to improvements in progression-free survival or overall survival.²

While there is no “one-size-fits-all” answer to the cost challenges facing consumers, we believe an important first step is increased transparency in several key areas of the pharmaceutical industry, including: more clarity and information around pricing, research and development costs; the process of label development; and an outcomes data to better understand the treatment's value. Such transparency will help ensure that targeted, effective policy solutions can be pursued.

We also recommend advancing a multi-pronged approach to addressing costs, as the introduction of new market competitors for single-source drugs will not be effective if done in a vacuum. One important area in this regard involves the Medicaid rebate program. To participate in the Medicaid program, pharmaceutical manufacturers must provide a specific discount to states and the federal government for most innovator drugs that equals the greater of either: (1) a 23.1% discount off of the drug's average manufacturer price (AMP); or (2) the difference between the AMP and the best price obtained in the private market. Of specific concern, this “best price” policy encourages drug manufacturers participating in Medicaid to set launch prices higher than what they might otherwise be in a competitive market in order to limit the impact of providing these discounts to the Medicaid population. Policymakers should consider options to level the playing field so that market forces can work to lower the cost of drugs for both public and private payers.

Aligned with these policies, health plans have ongoing efforts to utilize innovative benefit designs that recognize the unique nature of specialty drugs. By covering specialty drugs for their intended uses and monitoring the effectiveness, including side effects, health plans help ensure that individuals receive safe, high-value care that is also affordable.

2) Do the payers in the programs have adequate information to know the cost, patient volume, and increases in efficacy of a new treatment regimen?

As noted in the Committee's report, “If the payers do not have the opportunity to know what is coming and react accordingly with their plans and pricing, that is a problem. The

² Sham Mailankody, MB BS¹; Vinay Prasad, MD, MPH Five Years of Cancer Drug Approvals Innovation, Efficacy, and Costs. JAMA Oncology, July 2015.
<http://oncology.jamanetwork.com/article.aspx?articleid=2212206>

Committee should explore ways to provide greater transparency in this area.” We agree with the Committee’s conclusion and strongly support efforts to increase transparency in this area, particularly for drugs approved through expedited pathways.

For example, the FDA does not make information publicly available about the specific drugs that have been granted breakthrough status. This information is made public only if the manufacturer itself decides to disclose such information. Moreover, the FDA only posts the number of requests received for breakthrough designation and how many of these requests have been approved, denied, or withdrawn.³ There is no information (not even summary information) about the specific drugs, targeted disease(s), or patient population, let alone efficacy as compared to existing treatments. These are all critical pieces of information for providers, payers, and consumers. In addition, there is virtually no information about pricing, which adds to serious challenges facing health plans when assessing the costs and benefits of these potential treatments for the recommended patient populations.

The need for greater transparency cannot be overstated. A 2015 study by Avalere Health provided a snapshot of the government spending impacts of ten breakthrough medications. Their analysis found that just these ten drugs alone will cost federal and state governments *nearly \$50 billion over a decade* due to spending in Medicare, Medicaid and exchange plans. This soaring price tag represents a fraction of the total cost of future treatments for patients and taxpayers, as these ten drugs represent a small subset of the more than 5,400 medications in the drug pipeline. This figure also does not include costs to private payers or other government programs.⁴ Given the significant cost impact of just 10 drugs, it is even more critical that we have a comprehensive understanding of the broader universe of therapies that will impact consumers, payers, and the entire health care system in the coming years.

We recognize that efforts to increase transparency will likely be met with concerns by the pharmaceutical industry about revealing such information because it may include sensitive or proprietary information. Certainly, meaningful steps toward transparency can be taken that balance these concerns with the interests of all stakeholders. In fact, many companies issue press releases announcing expedited review of various therapies, such as breakthrough designation. However, the details offered in such releases vary greatly. Initial steps could involve having HHS or another entity provide summaries of projected approvals or trends for the upcoming year, including how many drugs are seeking expedited approval in different therapeutic areas and related statistics on potential

³ FDA will not disclose information regarding sponsors who submitted requests for or who have been granted or denied breakthrough therapy designation. Breakthrough therapy designation requests are typically submitted to an IND, and the FDA cannot disclose the existence of an IND, or any submissions that have been submitted to the IND, unless it has previously been publicly disclosed or acknowledged per 21 CFR 312.130(a).

<http://www.fda.gov/RegulatoryInformation/Legislation/SignificantAmendmentstotheFDCA/FDASIA/ucm341027.htm>

⁴“An Analysis of the Impact of Breakthrough Therapies on Government Spending.” June 8, 2015. <http://avalere.com/expertise/managed-care/insights/the-future-cost-of-innovation-an-analysis-of-the-impact-of-breakthrough-the>

populations. Additional updates could add related information of interest, including pricing, potential impact on public programs, as well as effectiveness compared to existing therapies. This is an approach that could be built and adapted over time as needed. Regarding clinical data, another approach would be to require that all pertinent information has been submitted to clinicaltrials.gov prior to the FDA accepting a manufacturer's NDA and that summaries of this information are made publically available.

Further, while use of expedited pathways, such as breakthrough designation, may serve to bring needed treatments to patients more quickly, such drugs are also being approved with different standards in terms of clinical evidence. Thus, we believe that increased transparency should not only include the areas highlighted above but also transparency about the different nature of the clinical evidence when expedited approval pathways are used and what it means (for example, qualifications or requirements to be approved as a breakthrough therapy). A robust discussion of the key issues in this regard was highlighted in a 2014 article in the *New England Journal of Medicine*.⁵ These issues include: implications when approval is based on preliminary evidence and surrogate endpoints, including limited profile information on a therapy's risks and benefits, and implications for both providers and patients when they are making treatment decisions.

3) What role does the concept of “value” play in this debate, and how should an innovative therapy’s value be represented in its price?

According to the Committee’s report, “The Committee should consider that cost, patient volume, and increases in efficacy ultimately speak to the concept of value.” The report further states “the Committee should turn its attention to ensuring that the program is getting value for the spending in Part D,” and “the Committee will also have to consider whether the payers in Medicare and Medicaid are doing enough to ensure that innovative drugs produce additional value that supports their additional expense.”

We agree that paying for value is critical given how the report and recent findings by the Medicare Trustees demonstrate the increasing threat drug pricing policies have on the continuing viability of the public programs. Health plans have assumed a leading role in advancing solutions that promote greater value for patients. For example, plans employ Pharmacy & Therapeutics (P&T) Committees to review and assess the latest medical evidence and determine the efficacy and value of new prescription drug products. A recent study demonstrates health plans are delivering greater value for consumers by integrating medical and prescription drug benefits, which contribute to significantly lower drug costs when these benefits are included in Medicaid health plan benefit packages.⁶ However, there are significant barriers as a result of statutory and regulatory requirements noted below that may slow or inhibit the development of pay for performance and other types of contracting arrangements in public programs.

⁵Darrow et al. “New FDA Breakthrough-Drug Category — Implications for Patients.” *New England Journal of Medicine*. March 27, 2014.

⁶ The Menges Group, “Comparison of Medicaid Pharmacy Costs and Usage in Carve-In Versus Carve-Out States”, April 2015.

Our members are keenly focused on developing potential solutions to overcome these barriers. A recent proposal included in the Administration's FY2017 Budget to establish a "coverage with evidence" process for Medicare Part D would be an important first step. Our understanding is this proposal would require the program to perform additional evidence-based reviews to determine coverage policy, including manufacturer-funded clinical trials focused on the Medicare population. These activities would significantly add to the information available to Part D plans as they establish their coverage policies.

We also support additional flexibility that would allow Part D plans to make formulary changes during the plan year as new information becomes available. Existing CMS policies permit these changes when they are "positive," or adding to the scope of coverage, but limit Part D plans from establishing additional management tools except when safety issues are identified. These tools -- which have already proven to be a success in the commercial market -- should be permitted in the Part D program. As we note below, we have similar concerns about lack of flexibility for Medicaid health plans to use these clinically-based tools to support value and cost-effectiveness for beneficiaries and states.

In addition to these areas, we have identified several other steps that can be taken to better promote the concept of value in coverage policy for Part D and Medicaid.

- One of the conditions for coverage under Part D and Medicaid is that a drug or treatment is being used for a medically-accepted indication. Such indications include both uses approved by the FDA as well as off-label uses referenced in certain compendia.⁷ CMS guidance specifies that Part D sponsors reference all CMS recognized compendia to determine whether there are any supportive citations prior to determining that a drug is not being used for a medically-accepted indication.⁸ However, it is our understanding there are no conflict of interest limitations on the individuals working for these compendia except when reviewing anti-cancer drugs. These compendia and the guidelines should disallow individuals with financial relationships with a pharmaceutical manufacturer or other relevant party from participating in decisions involving that entity. Further, the decision-making processes for these organizations should be public and include opportunities for outside entities to provide input.
- Federal law on Medicaid "best price" should not discourage innovative payment models between payers and pharmaceutical makers. Payers and manufacturers are considering arrangements where the payer would reimburse the manufacturer a base rate with an additional reimbursement if the drug proves to be an effective treatment for the patient. The Medicaid "best price" law may create a disincentive to enter into such value-based models.

⁷ Section 1860D-2(e) cross-referencing Sections 1927(k)(6) and 1927(g)(1)(B)(i).

⁸ See Chapter 6, Section 10.6 of the Medicare Prescription Drug Benefit Manual.

- CMS should identify and address potential barriers to establishing value-based arrangements between plans and manufacturers. For example, we understand many of these arrangements include rebates for performance over a multiyear period. However, the Part D plan bidding tool does not permit the reporting of multi-year rebates to determine allowable costs. CMS, Part D plans, and other stakeholders should engage in a collaborative process to identify other barriers to value-based arrangements that may exist and steps to address them.
- We remain concerned that federal requirements that limit Part D plan management tools are antithetical to extending the value proposition. For example, the CMS protected classes policy restricts the use of clinically-based management tools that promote the more effective use of prescription drugs for individuals with specified conditions. Similarly, CMS' interpretation of the federal law mandating Part D plans cover at least two drugs in every prescription drug class is inconsistent with program goals. Our member plans develop management tools with the advice and consent of independent health care practitioners serving on P&T Committees, and Part D law permits opportunities for beneficiaries to seek exceptions for a drug not included on a plan's formulary. The additional limits put in place by the protected class and two-drug requirements are therefore unnecessary and counterproductive as we seek to promote greater value in the Part D program.
- Finally, we strongly support increased transparency by pharmaceutical manufacturers to promote a greater understanding of their true costs.

4) What measures might improve price transparency for new higher-cost therapies while maintaining incentives for manufacturers to invest in new drug development?

We strongly believe that increased transparency is a critical element in addressing the significant challenges associated with high cost drugs. This involves transparency not only regarding the price of the drug itself but key factors contributing to a drug's price, including:

- Pre-approval: estimated price, cost of a course of treatment, each label iteration and projected government spending;
- Post-approval: annual reporting of price increases as well as the number of times a price was increased during the year; and
- Research and development costs incurred by the company versus the amount of such costs incurred by other private or public entities. This information could be provided at time of filing for FDA approval and could be updated annually.

Greater transparency in prescription drug pricing is aligned with the push for transparency in other facets of the health system. Under the Physician Payments Sunshine Act provisions of the ACA, manufacturers of drugs, medical devices, and biologicals that participate in federal health care programs are required to track and report certain

payments and items of value given to physicians and teaching hospitals. The ACA also required health plan issuers with rate increases above 10% to submit a justification to the government for review and make summary information accessible to the public in an understandable format.

Prescription drug prices and costs are rising at an alarming pace, and the problem of crippling drug price increases is far more widespread than a few “bad actors.” Medicare’s new Drug Dashboard provides clear evidence of this fact. The dashboard captures drugs that rank towards the top either in terms of overall spending or price increases. Ten drugs in the dashboard had price increases of 20% or more from 2013-2014. These drugs accounted for \$7.5 billion in Medicare spending and were used by nearly 7 million beneficiaries. Thirty drugs on this list had price increases of 7% or more from 2013-2014, accounting for \$30 billion in Medicare spending and were used by nearly 14 million beneficiaries.

These figures are simply staggering and illustrate the significant impact high cost pharmaceuticals are having on patients, payers, and taxpayers. Given the enormous costs of current and future medications, we believe transparency is the first step towards understanding what future policy solutions may lead to are needed to ensure access to affordable medications. We are not advocating for price controls. Rather, we are advocating for the gathering of essential information so that policymakers can better understand the issue and develop solutions to help ensure the market is functioning properly.

5) What tools exist, or should exist, to address the impact of high cost drugs and corresponding access restrictions, particularly on low-income populations and state Medicaid programs?

The Committee’s report documents increasing costs faced by state Medicaid programs due to pharmaceutical pricing practices. In addition to these findings, a recent Avalere report⁹ estimated federal and state governments would face almost an additional \$16 billion in Medicaid spend in the next ten years due to the introduction of just ten new products. With increasing numbers of prescription drugs in the pipeline that will likely be launched at very high prices, we agree additional steps should be taken to ensure Medicaid programs are receiving the best value for their prescription drug dollars.

Medicaid health plans play a key role in supporting the ongoing viability of the Medicaid program while ensuring beneficiaries have access to the breakthrough medications they need. A recent study¹⁰ finds prescription drug costs in states which include pharmacy and medical benefits in Medicaid health plan coverage were 14.6% lower (generating an

⁹Avalere. “An Analysis of the Impact of Breakthrough Therapies on Government Spending.” June 8, 2015. <http://avalere.com/expertise/managed-care/insights/the-future-cost-of-innovation-an-analysis-of-the-impact-of-breakthrough-the>

¹⁰ The Menges Group, “Comparison of Medicaid Pharmacy Costs and Usage in Carve-In Versus Carve-Out States”, April 2015.

estimated savings of \$2 billion in 2014) than in states which maintained fee-for-service coverage for medications by a “carve out” from Medicaid health plan benefits. These savings are often achieved through Medicaid health plan clinically-based management tools including generic substitution and formulary management techniques promoting the use of the most appropriate, cost-effective medications.

However, CMS has recently signaled it may limit Medicaid health plan use of these tools. The Social Security Act guarantees coverage of prescription drugs manufactured by companies participating in the Medicaid Prescription Drug Rebate programs subject to prior authorization programs operated according to specified rules. It is our understanding states vary in the flexibility provided to Medicaid health plans to design clinically-based programs consistent with commercial market practices. The regulation proposed by CMS last year to significantly modify rules governing Medicaid health plans included a requirement that Medicaid health plans provide coverage of prescription drugs to comply with standards of coverage defined in the Act.¹¹ We strongly suggest the final rule make clear that Medicaid health plans can maintain control of their tools that are promoting the use of clinically appropriate, cost effective medications.

Our recommendations above to support value in the Part D program are also relevant to Medicaid. Federal law and regulations should ensure the compendia directed by the law to determine Medicaid prescription drug coverage policy, the organizations developing the standards for off-label use, and state Medicaid P&T Committees comply with strict conflict of interest standards. The “coverage by evidence” methods proposed for Part D should be incorporated in the agreements signed by manufacturers pursuant to the Medicaid Prescription Drug Rebate Program, as should a requirement to report R&D costs for all covered drug products. We strongly believe these recommendations would increase the value proposition in the Medicaid program while preserving access to appropriate breakthrough medications.

¹¹ See proposed 438.3(s), 80 FR 31098 at p. 31257.